

Characterization of Human Skeletal Muscle Stem Cells for Clinical Application

Grant Award Details

Characterization of Human Skeletal Muscle Stem Cells for Clinical Application

Grant Type: New Faculty Physician Scientist

Grant Number: RN3-06504

Project Objective: To develop approaches to characterize and transplant endogenous hMuSC and to perform pre-clinical studies that will provide the basis for IND-enabling studies to develop hMuSC as a therapeutic. A specific target disease has not been specified. In consideration are muscle ailments that present an important unmet clinical need but only affect small isolated muscles that could be regenerated with localized transplantation of relatively small numbers of cells. Examples include: 1) atrophy and muscle dysfunction in the face or hand after nerve injury or direct trauma that does not recover after reinnervation. 2) extraocular muscle injury causing inability to move the eye and diplopia seen in Graves' disease, traumatic injury, oculopharyngeal muscular dystrophy and progressive external ophthalmoplegia. 3) urinary and fecal incontinence, for which therapies that address the small sphincter muscles involved have great potential.

Investigator:

Name: Jason Pomerantz
Institution: University of California, San Francisco
Type: PI

Disease Focus: Skeletal/Smooth Muscle disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: \$3,084,000

Status: Active

Progress Reports

Reporting Period: Year 1

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Reporting Period:	Year 3
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Reporting Period:	Year 5
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Grant Application Details

Application Title:	Characterization of Human Skeletal Muscle Stem Cells for Clinical Application
Public Abstract:	<p>Skeletal muscle makes up 40% of our bodies, dictates our form, is responsible for our ability to move, express ourselves, eat, breath and to look around. Restoration or preservation of the body's normal form and function is the central goal of regenerative medicine and the central focus of my clinical specialty of plastic and reconstructive surgery. Skeletal muscle injury and disease are major sources of morbidity that affect millions. The potential for treating these ailments with regenerative approaches that use stem cells is enormous because skeletal muscle naturally has its own stem cell population. However, to make human muscle stem cells a usable clinical tool we need to be able to characterize them, transplant them, and induce them to function effectively. To accomplish these goals, our objectives are to identify appropriate human muscles to harvest stem cells from, to develop techniques to transplant them, and to test them in models of diseases. This work is expected to lead directly to treatments for patients with several disabling muscle degenerative conditions that affect small but critical muscles that could be regenerated with localized muscle stem cell transplants. Examples include facial paralysis or loss of hand function, and conditions affecting the eye muscles causing vision loss. This work also lays the groundwork for tissue engineering of muscles, and will contribute to approaches to correct genetic muscle defects or treat other systemic muscle diseases.</p>
Statement of Benefit to California:	<p>Skeletal muscle injury and disease are major sources of morbidity that affect Californians of all ages, including the veteran population. Regenerative medicine and stem cell biology offer great potential for opportunities to improve upon current treatments and to develop approaches for many of the muscle ailments that remain essentially untreatable. Skeletal muscle stem cells have been well characterized in mice, and preliminary evidence suggests similar approaches in humans will enable clinical translation. Therefore, the proposed research seeks to develop stem cell therapies that will directly impact muscle ailments that are at the root of diverse deformities and disabilities of the face, body and limbs. California is the birthplace of many of the great advances in reconstructive surgery, including microsurgery and muscle flaps, largely through the support of its people, and their pioneering spirit. In turn, Californians have benefited and continue to benefit from the newest and best approaches that reconstructive surgery has to offer. We propose to continue to improve reconstructive and regenerative options for the citizens of California by building on our strong historical foundation to address current needs. Our research will carry on this tradition because of our focus on problems of form and function affecting many of our citizens, the logical path to muscle stem cell clinical application, and the unique clinical and scientific focus and potential of our team.</p>